European Medicines Agency

EMEA/H/C/157

EUROPEAN PUBLIC ASSESSMENT REPORT (EPAR)

CEREZYME

EPAR summary for the public

This document is a summary of the European Public Assessment Report (EPAR). It explains how the Committee for Medicinal products for Human Use (CHMP) assessed the studies performed, to reach their recommendations on how to use the medicine.
If you need more information about your medical condition or your treatment, read the Package Leaflet (also part of the EPAR) or contact your doctor or pharmacist. If you want more information on the basis of the CHMP recommendations, read the Scientific Discussion (also part of the EPAR).

What is Cerezyme?

Cerezyme is a powder to be made up into a solution for infusion (a drip into a vein). Cerezyme contains the active substance imiglucerase.

What is Cerezyme used for?

Cerezyme is used as long-term enzyme replacement therapy in patients with Gaucher disease. Gaucher disease is a rare inherited disorder. People with the disease do not have enough of an enzyme, glucocerebrosidase. This enzyme normally breaks down a fatty waste product called glucocerebroside. Without the enzyme, glucocerebroside builds up in the body, typically in the liver, spleen and bone marrow, and this produces the symptoms of the disease: anaemia (low blood count), tiredness, easy bruising and a tendency to bleed, an enlarged spleen and liver, and bone pain and fractures.
Cerezyme is used in patients who have type 1 (non-neuronopathic) or type 3 (chronic neuronopathic) Gaucher disease and who have one or more of the following conditions caused by the disease:

- anaemia (low red blood cell counts),
- thrombocytopenia (a decrease in the number of platelets in the blood),
- bone disease,
- hepatomegaly (enlarged liver) or splenomegaly (enlarged spleen).

The medicine can only be obtained with a prescription.

How is Cerezyme used?

Cerezyme should be given by doctors experienced in the management of patients with Gaucher disease. It is given by infusion over one to two hours every two weeks with higher doses at first. The doses are then adjusted according to how the patient responds to the treatment.
Cerezyme is designed for long-term use.

How does Cerezyme work?

Gaucher disease has previously been treated using an enzyme, alglucerase, prepared from human placentae. Imiglucerase, the active substance in Cerezyme, is a copy of this enzyme, produced by a method known as 'recombinant DNA technology': the enzyme is made by a cell which has received a gene (DNA) that makes it able to produce the enzyme. Imiglucerase helps (catalyses) the break down of glucocerebroside and stops it building up in the body.

7 Westferry Circus, Canary Wharf, London E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 74 18 84 16
E-mail: mail@emea.europa.eu http://www.emea.europa.eu

How has Cerezyme been studied?
For type 1 Gaucher disease, Cerezyme has been studied in three studies, and in a total of 40 patients (this is an acceptable number because the disease is very rare). The studies examined Cerezyme's effectiveness in controlling the symptoms of Gaucher disease, such as normalising (increasing) the number of red blood cells and platelets in the blood, and normalising (decreasing) the size of the liver and spleen. Cerezyme was compared to alglucerase. For type 3 Gaucher disease (which is an extremely rare form of the disease), data from published articles and from the special register of Gaucher's patients were used.

What benefit has Cerezyme shown during the studies?
The studies have shown that Cerezyme is as safe and effective as alglucerase in controlling the symptoms of Gaucher disease. It has also been shown that patients may safely switch from alglucerase to imiglucerase treatment.

What is the risk associated with Cerezyme?
The most common side effects with Cerezyme (seen in between 1 and 10 patients in 100) are respiratory symptoms, urticaria or angioedema (hives), pruritus (itching) and rash. Patients can develop antibodies (proteins that are produced in response to Cerezyme and can affect treatment) and they should be monitored for any allergic reaction to Cerezyme during the first year of treatment. Cerezyme should be used carefully in people who may be hypersensitive (allergic) to imiglucerase or any of the other ingredients.

Why has Cerezyme been approved?
The Committee for Medicinal products for Human Use (CHMP) decided that Cerezyme gives effective control of the symptoms of types 1 and 3 Gaucher disease. The Committee decided that Cerezyme's benefits are greater than its risks and recommended that Cerezyme be given marketing authorisation.

Other information about Cerezyme:
The European Commission granted a marketing authorisation valid throughout the European Union for Cerezyme to Genzyme Europe B.V. on 17 November 1997. The marketing authorisation was renewed on 17 November 2002 and on 17 November 2007.

The full EPAR for Cerezyme can be found here.

This summary was last updated in 11-2007.